Ad hoc announcement pursuant to Art. 53 LR

Santhera and ReveraGen Complete NDA Submission to FDA for Vamorolone in Duchenne Muscular Dystrophy

Pratteln, Switzerland, and Rockville, MD, USA, October 27, 2022 – Santhera Pharmaceuticals (SIX: SANN) and ReveraGen BioPharma, Inc announce that they have completed the rolling submission of a new drug application (NDA) to the U.S. Food and Drug Administration (FDA), seeking priority review for vamorolone for the treatment of Duchenne muscular dystrophy (DMD). Subject to approval, vamorolone is set to become available to patients in the U.S. in H2-2023.

At the core of the NDA submission are positive data from the pivotal Phase 2b VISION-DMD study which comprised (1) a 24-week period to demonstrate efficacy and safety of vamorolone (2 and 6 mg/kg/day) versus prednisone (0.75 mg/kg/day) and placebo, followed by (2) a 24-week period to evaluate the maintenance of efficacy and collect additional longer-term safety and tolerability data [1]. In addition, the filings include data from four open-label studies (including extension) in which vamorolone was administered at doses between 2 and 6 mg/kg/day for a total treatment period of up to 30 months [2] and an external comparator study.

“Completion of the vamorolone NDA submission is a major step towards our goal of bringing this investigational therapy to patients living with DMD, and represents a vital milestone for Santhera,” said **Dario Eklund, CEO of Santhera**. “We look forward to working closely with U.S. regulators to advance vamorolone towards approval.”

“We would like to express our deepest gratitude to the study participants, their families and caregivers, as well as the investigators and study staff. Their unwavering commitment enabled us to achieve this milestone,” said **Eric Hoffman, PhD, President and CEO of ReveraGen BioPharma**. “If approved, vamorolone will emerge as an addition to current standards of care in DMD with the potential to address unmet medical needs and treat a majority of Duchenne patients starting at an early age.”

With the completion of the rolling NDA submission, Santhera and ReveraGen have also applied for priority review. Typically, within 60 days of the receipt of the dossier, the FDA will inform if a priority review will be granted. A priority review designation indicates FDA’s goal is to take action on an application within six months (compared to ten months under standard review) which would set an anticipated approval date for as early as mid-2023.

In Europe, Santhera has submitted a marketing authorization application (MAA) to the European Medicines Agency (EMA) in September 2022, seeking approval for vamorolone in the European Union, as well as in Norway, Liechtenstein and Iceland.

Vamorolone has been granted Orphan Drug status in the U.S. and in Europe for DMD, and has received Fast Track and Rare Pediatric Disease designations by the U.S. FDA and Promising Innovative Medicine (PIM) status from the UK MHRA for DMD.

About Vamorolone

Vamorolone is an investigational drug candidate with a mode of action based on binding to the same receptor as corticosteroids but modifying its downstream activity and as such is considered a dissociative anti-inflammatory drug [2-5]. This mechanism has shown the potential to 'dissociate' efficacy from steroid safety concerns and therefore vamorolone could emerge as an alternative to existing corticosteroids, the current standard of care in children and adolescent subjects with DMD. In the pivotal VISION-DMD study, vamorolone met the primary endpoint Time to Stand (TTSTAND) velocity versus placebo (p=0.002) at 24 weeks of treatment and showed a good safety and tolerability profile [1]. The most commonly reported adverse events versus placebo from the VISION-DMD study were cushingoid features, vomiting and vitamin D deficiency. Adverse events were generally of mild to moderate severity. Vamorolone is an investigational medicine and is currently not approved for use by any health authority.

References:
[1] Guglieri M et al (2022). JAMA Neurol. Published online August 29, 2022. doi:10.1001/jamaneurol.2022.2480. Link.
[2] Mah JK et al (2022). JAMA Netw Open. 2022;5(1):e2144178. doi:10.1001/jamanetworkopen.2021.44178. Link.
[3] Guglieri, et al (2022) JAMA. doi:10.1001/jama.2022.4315
[4] Heier CR at al (2019). Life Science Alliance DOI: 10.26508
[5] Liu X, et al (2020). Proc Natl Acad Sci USA 117:24285-24293

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is a rare inherited X-chromosome-linked disease, which almost exclusively affects males. DMD is characterized by inflammation which is present at birth or shortly thereafter. Inflammation leads to fibrosis of muscle and is clinically manifested by progressive muscle degeneration and weakness. Major milestones in the disease are the loss of ambulation, the loss of self-feeding, the start of assisted ventilation, and the development of cardiomyopathy. DMD reduces life expectancy to before the fourth decade due to respiratory and/or cardiac failure. Corticosteroids are the current standard of care for the treatment of DMD.

About Santhera

Santhera Pharmaceuticals (SIX: SANN) is a Swiss specialty pharmaceutical company focused on the development and commercialization of innovative medicines for rare neuromuscular and pulmonary diseases with high unmet medical need. The Company has an exclusive license for all indications worldwide to vamorolone, a dissociative steroid with novel mode of action, which was investigated in a pivotal study in patients with Duchenne muscular dystrophy as an alternative to standard corticosteroids. Santhera has submitted a new drug application (NDA) to the U.S. FDA and a marketing authorization application (MAA) to the European Medicines Agency (EMA) for vamorolone for the treatment of DMD. The clinical stage pipeline also includes lonodelestat to treat cystic fibrosis (CF) and other neutrophilic pulmonary diseases. Santhera out-licensed rights to its first approved product, Raxone® (idebenone), outside North America and France for the treatment of Leber's hereditary optic neuropathy (LHON) to Chiesi Group. For further information, please visit www.santhera.com.

Raxone® is a trademark of Santhera Pharmaceuticals.

About ReveraGen BioPharma

ReveraGen was founded in 2008 to develop first-in-class dissociative steroidal drugs for Duchenne muscular dystrophy and other chronic inflammatory disorders. The development of ReveraGen's lead compound, vamorolone, has been supported through partnerships with foundations worldwide, including Muscular Dystrophy Association USA, Parent Project Muscular Dystrophy, Foundation to

Eradicate Duchenne, Save Our Sons, JoiningJack, Action Duchenne, CureDuchenne, Ryan's Quest, Alex's Wish, DuchenneUK, Pietro's Fight, Michael's Cause, Duchenne Research Fund, and Defeat Duchenne Canada. ReveraGen has also received generous support from the US Department of Defense CDMRP, National Institutes of Health (NCATS, NINDS, NIAMS), and European Commission (Horizons 2020). www.reveragen.com

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